GAIN THERAPEUTICS

FACTSHEET | Q1 2024

Leading the Discovery of Allosteric Binding Sites & Creating New Medicines

We are a clinical-stage biotechnology company accelerating drug discovery and unlocking novel disease-modifying treatments. Deploying our highly advanced platform, we are identifying and targeting never before seen allosteric binding sites on disease-implicated proteins for diseases that are hard-to-treat or for which there are no current disease-modifying therapies.

Our Magellan™ Proprietary Platform

Leveraging AI-supported structural biology, proprietary algorithms and supercomputer-powered physics-based models, our Magellan discovery platform can identify novel allosteric binding sites on proteins, pinpointing pockets that cannot be found or drugged with current technologies. Magellan allows us to create new medicines that activate, inhibit, stabilize, destabilize and degrade proteins based on disease biology.

Lead Program

Borne of our proprietary platform, our lead program GT-02287 is targeting GBA1-Parkinson's Disease (GBA1-PD). GT-02287 is a an oral, brain penetrant small molecule allosteric modulator that restores the function of the lysosomal protein enzyme glucocerebrosidase (GCase) which becomes misfolded and impaired to a GBA1 gene mutation, the most common genetic abnormality associated with PD. In preclinical models of PD, GT-02287 restored GCase enzymatic function, reduced aggregated α-synuclein, neuroinflammation and neuronal death, increased dopamine levels, improved motor function and significantly reduced plasma neurofilament light chain (NfL) levels, an emerging biomarker for neurodegeneration.

Our Pipeline

INDICATION	TARGET	DISCOVERY	RESEARCH	PRECLINICAL	PHASE 1	PHASE 2
GBA1-Parkinson's Disease Idiopathic Parkinson's Disease	GCase					
Dementia with Lewy bodies Alzheimer's Disease	GCase					
Gaucher Disease	GCase					
GM1 Gangliosidosis	GLB					
Krabbe Disease	GALC					
Alpha-1 Antitrypsin Deficiency	A1AT					
Solid Tumors	TO BE DISCLOSED					
Solid Tumors	TO BE DISCLOSED					

Neurodegeneration · Lysosomal Storage Disorders · Metabolic Disorders · Oncology

RECENT HIGHLIGHTS

Initiation of Phase I clinical trial for lead program GT-02287 for GBA and idiopathic Parkinson's disease

Expansion of clinically validated AI-drug discovery platform now accesses chemical space in excess of 50 billion compounds

Presentation of novel preclinical data at International Congress of Parkinson's Disease and Movement Disorders® showing positive effect of lead candidate GT-02287 on multiple facets of Parkinson's disease

Awarded 2.5M CHF grant to advance lead program in Parkinson's disease

KEY UPCOMING MILESTONES

Completion of Phase I clinical trial in healthy subjects

Initiation of first-in-patient clinical study in patients with GBA and idiopathic Parkinson's disease

Interim proof-of-concept biomarker readout in patients

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